Project Report

Patient-Engaged Health Technology Assessment Strategy

Feasibility Assessment and Recommendations

Zachary S. Predmore, Thomas W. Concannon, Lori Frank

RAND Health Care

PR-A2091-1
August 2022
Prepared for the Innovation and Value Initiative

About This Project Report

Methods for collecting patient input on the value of health care interventions are in wide use, but the extent to which existing methods capture the full range of outcomes important to patients has not been established. There is no standard approach to systematically identify and quantify patient-important outcomes for use in deliberative decision-making processes.

This report summarizes a newly proposed method for engaging patients in health care valuation, using information from patient framing of goals for treatment. The report documents the approach we took to establish its feasibility for wider use. We offer a feasibility assessment and recommendations on how to adopt the strategy in health technology assessment, using the disease state of rheumatoid arthritis as an example.

RAND Health Care

RAND Health Care, a division of the RAND Corporation, promotes healthier societies by improving health care systems in the United States and other countries. We do this by providing health care decisionmakers, practitioners, and consumers with actionable, rigorous, objective evidence to support their most complex decisions. For more information, see www.rand.org/health-care, or contact:

RAND Health Care Communications
1776 Main Street
P.O. Box 2138
Santa Monica, CA 90407-2138
(310) 393-0411, ext. 7775
RAND_Health-Care@rand.org

Acknowledgments

We are grateful for the support of our funders of this project at the Innovation and Value Initiative (IVI), including Jennifer Bright, Richard Chapman, PhD, and Richard Xie, PhD. We are also grateful for the help from our Project Steering Committee, whose members include: Susan Bartlett, PhD, of McGill University; Clifton Bingham, MD, of Johns Hopkins University; Deb Constien, patient representative; Stacy Courtnay, patient representative; Julie Eller of the Arthritis Foundation; Erin Giovanetti of Medstar Health; Juan Marcos Gonzalez, PhD, of Duke University School of Medicine; R. Brett McQueen, PhD, of University of Colorado Anschutz Medical Campus; Lawrence “Rick” Phillips, EdD, patient representative; Suz Schrandt of ExPPect LLC.

We are also grateful for the contributions of our RAND Corporation internal reviewers, including Carrie Farmer, PhD, Paul Koegel, PhD, and Claire O'Hanlon, PhD.

Summary

Commonly used approaches to assessing the value of health technologies fail to capture a comprehensive set of clinical and economic outcomes that matter to patients and their caregivers. No standard approach is available to identify and quantify patient-important outcomes with a focus on their use in health care valuation processes that inform health technology assessment (HTA), a systematic approach that many governments and payers use to estimate the value of health technologies.

We developed the Patient-Engaged Health Technology Assessment Strategy to address this need. This strategy uses principles of goal attainment scaling (GAS) to frame survey-based goal collection from patients to yield output suitable for incorporation into multi-criteria decision analysis (MCDA) or other deliberative methods.

Through empirical work, we found that goals relevant for treatment evaluation and comparison can be efficiently identified and rated for importance by a patient population. Patient-important goals can be incorporated into deliberative health care valuation using this method to permit wide input from patients with the lived experience of disease. Deliberative methods of valuation can include outcomes based on goals collected directly from patients. Patient input through goal framing provides a way for patients to be actively involved in valuation methods.

This report describes the approach we took to explore the feasibility of this strategy, and it offers an assessment of the feasibility of implementing the strategy in HTA, as well as several recommendations for implementation.

Contents

Figures and Tables

Figures

Tables

Patient-Engaged Health Technology Assessment

Background

Incorporating patient-important outcomes into health research and care is a core principle of patient-centered medicine (Epstein & Street, 2011; Frank, Basch, Selby, & Institute, 2014). However, patient-centered principles have yet to reach some topics and methods in health research. Commonly used approaches to assessing the value of health technologies, such as the cost per quality-adjusted life year (QALY), fail to capture a comprehensive set of clinical and economic outcomes that matter to patients and their caregivers (Garrison, Jansen, Devlin, & Griffin, 2019; Perfetto, 2018). To date, we lack a standard approach to identify and quantify patient-important outcomes in a way that would make the measures appropriate for use in deliberative processes such as multi-criteria decision analysis (MCDA).

Definition of Health Technology Assessment[5]

"a multidisciplinary process that uses explicit methods to determine the value of a health technology ... to inform decision-making in order to promote an equitable, efficient, and high-quality health system."

Formal HTA is expanding in the United States with consequences for coverage of and access to health interventions (O'Rourke, Oortwijn, & Schuller, 2020). Inputs incorporated into existing HTA consist primarily of data on clinical efficacy or effectiveness of the health technologies being assessed and associated health-related costs (Thokala, Carlson, & Drummond, 2020). While these data are essential, treatment attributes and impacts beyond clinical effectiveness are often of high value to patients, clinicians, and health systems as they make treatment decisions; however, they are often excluded from HTA. Failure to consider a broader set of patient-important outcomes in HTA might lead to incomplete assessments of the value of health technologies and decisions, ultimately impacting the welfare of patients and resource allocation efficiency. Patient input is often considered supplemental rather than central to existing HTA approaches (Facey & Single, 2017). The usefulness of HTA is also limited when measured benefits of treatments are based on an "average" patient or on population samples not representative of heterogeneous patient experiences in the real world.

What is included and who is consulted in HTA have been the subjects of much discussion recently, and some have opted to include more patient input in some parts of HTA processes. In a 2017 survey of patient advocates in eight countries, respondents from five countries indicated they were involved with HTA, often at the appraisal stage by submitting materials regarding

health technologies (Scott & Wale, 2017). In 2021, the United Kingdom's National Institute for Health and Care Excellence (NICE) released new guidance on how patient organizations can be meaningfully involved in different stages of HTA (Rasburn, Livingstone, & Scott, 2021). Methods for collecting patient input have also matured to reflect the diversity of patient preferences. For instance, Devlin and coauthors found that directly eliciting personal utility functions from the general population in the United Kingdom was feasible; this method is an important alternative to revealed preference methods wherein researchers infer patient preferences based on real-world choices (Devlin, Shah, Mulhern, Pantiri, & van Hout, 2019). Fraenkel and coauthors identified three distinct "preference phenotypes," or clusters of shared, distinct preferences among patients with rheumatoid arthritis (RA), and demonstrated that these phenotypes can be applied in shared decision-making, suggesting that patient values can be summarized at scale (Fraenkel, Nowell, Michel, & Wiedmeyer, 2018).

What is the Patient-Engaged Health Technology Assessment Strategy?

The Patient-Engaged Health Technology Assessment strategy involves two related and complementary aspects that can improve uptake of patient-centered principles in current approaches to HTA: 1) collecting outcomes that matter to patients and their caregivers (Lakdawalla et al., 2018) through survey methods based on the principle of goal attainment scaling (GAS) (Jennings, Ramirez, Hays, Wenger, & Reuben, 2018; Kiresuk & Sherman, 1968; Kiresuk, Smith, & Cardillo, 2014), and 2) involving patients with attention to representation across a range of backgrounds, along with other decision-makers (e.g., employers), through an inclusive process of deliberation such as Multi-Criteria Decision Analysis (MCDA) (Hansen & Devlin, 2019; Marsh, Goetghebeur, Thokala, & Baltussen, 2017; Oortwijn et al., 2022; Sanders et al., 2016). GAS is an approach commonly used to establish and evaluate treatment goals in shared decision-making in clinical practice. Goal attainment in clinical practice is a form of shared decision-making in which the clinician and patient follow progress on the patient's self-identified goals for treatment. GAS in our proposed approach is achieved through the development of a survey instrument for broad elicitation and rating of goals across a representative patient community. Elicited goals from the survey are rated for importance, and these rated goals may then be used to assess the value of different treatment options among members of the patient community in a patient-engaged HTA deliberation. While the collected and rated patient goals can be applied in a variety of deliberative methods for HTA, we focus on the application to MCDA in this report. MCDA is an inclusive deliberation process that has been adapted for use in HTA; it is an approach that engages different stakeholders impacted by the decision to identify, weigh, and apply multiple criteria to select the most preferred option(s) among multiple alternatives in a valuation or coverage decision made by an HTA body.

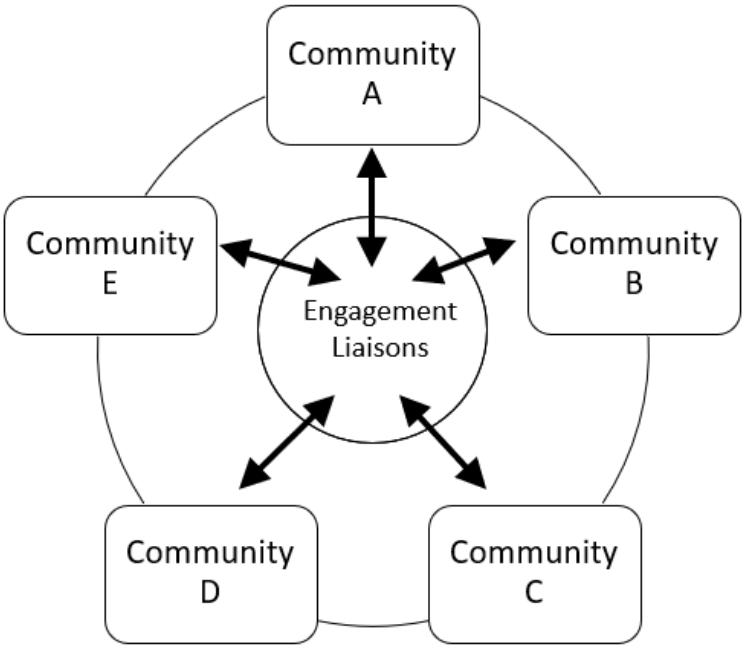

Figure 1. Hub-and-spoke model showing relationship between patient engagement liaisons and patient communities

In the Patient-Engaged Health Technology Assessment Strategy (Frank & Concannon, 2019), we propose an adaptation of a previously published MCDA model for use in health technology assessments: the MCDA value measurement model (Thokala et al., 2016). Our proposed adaptation adds patient engagement components (see Table 1), including a preliminary step, "Step 0," in which a set of patient-important goals is developed. Depending on the intended health technology question, the goals can be disease-specific and/or generic—that is, goals applicable across different disease areas. This step grounds the method in broad patient input. The adaptation also includes the engagement of patient liaisons who represent patient communities in a "hub-and-spoke" model (Figure 1). In this model, the term "alternatives" refers to the different treatment options that are available for a disease or condition.

Table 1. Steps in MCDA with proposed patient engagement components

Step	Description	Patient Engagement Components
0. Develop goal inventory	Develop a baseline inventory of goals appropriate for health technology question to be addressed. The inventory may include disease-specific and/or generic goals, for use within a single disease area and/or for use across	Engage a large patient panel to list, prioritize, and weight goals for their conditions. This should be a diverse group of patients with a variety of disease severities.

Step	Description	Patient Engagement Components
	multiple disease areas suitable for different MCDA exercises.	
1. Defining the decision problem	Identify objectives, type of decision, alternatives, stakeholders, and output required.	Include patient liaisons who represent the full spectrum of relevant patients.
2. Selecting and structuring criteria	Identify criteria relevant for evaluating alternatives.	Patient liaisons identify patient goal attainment statements from the inventory that are specific to technology alternatives for a specific condition.
3. Measuring performance	Gather data about the alternatives' performance on the criteria and summarize this in a "performance matrix."	Gather data on the performance of technology alternatives relative to patient-identified goals.
4. Scoring alternatives	Elicit stakeholders' preferences for changes within criteria.	Patient liaisons review and summarize data on patients' preferences for advancement toward goal attainment from the goal inventory in Step 0.
5. Weighting criteria	Elicit stakeholders' preferences between criteria.	Patient liaisons review and summarize data on patients' ratings of goal importance from the goal inventory in Step 0.
6. Calculating aggregate scores	Use the alternatives' scores on the criteria and the weights for the criteria to get "total value" by which the alternatives are ranked.	N/A
7. Dealing with uncertainty	Perform uncertainty [sensitivity] analysis to understand the level of robustness of the MCDA results.	N/A
8. Reporting and examination of findings	Interpret the MCDA outputs, including uncertainty analysis, to support decision-making.	Share, discuss, and obtain feedback from patients on MCDA outputs.

SOURCE: The "Step" and "Description" columns for steps 1 to 8 are taken from Thokala et al., 2016.

This project was designed to assess the feasibility of and develop recommendations for carrying out the patient-engagement components of this approach, referred to as the Patient-Engaged Health Technology Assessment Strategy. The strategy enhances the existing methods for the MCDA value measurement model and establishes how patient-centered goals in managing a disease condition through health interventions can be efficiently "crowd-sourced" from a large and representative patient population, and how these goals may be used as criteria and weights in an inclusive HTA deliberative process. Here we briefly describe the new patient engagement components of the model:

Step 0: Develop goal inventory. In this step, which takes place before any

deliberation phase of MCDA, patients would be surveyed to better understand their goals for their specific disease condition and their treatment(s). The initial set of goals specified in the survey instrument can be derived from existing literature and input from patient and clinician stakeholders. Write-in responses can also be included to allow additional goals to be added to the goal inventory (The sections below describe how we developed such a survey instrument in the disease state of rheumatoid arthritis.) This survey would be administered online to patients that are representative of the final target population for a proposed technology (including those representative of the range of demographic and socioeconomic characteristics relevant for the target population), and including patients across a range of disease severities or other clinical characteristics relevant for the technology's intended use. Online survey methods make collection of data from relatively large samples possible and make feasible the inclusion of patients across demographic and other variables of interest. For some conditions, for example in pediatric populations or for individuals with advanced cognitive impairment, researchers may also need to involve caregivers as proxies as well. To establish the goal inventory, patients would be asked to rate the importance of a set of goals and weight the importance of those goals. This step could include solicitation from the patient community of additional goals to include in an assessment. This goal inventory could then be used by patient liaisons in multiple future MCDA exercises.

Step 1: Defining the decision problem. In this step of identifying the objectives of the MCDA exercise, the proposed patient engagement component involves identifying and inviting patients to play a part in this process as liaisons to the broader patient community. The patients should be those affected by the condition being evaluated and should be broadly representative of the patient population in terms of demographics, disease severity, geography, etc.

Step 2: Selecting and structuring criteria. Drawing from the goal inventory developed in Step 0, the researchers and patient liaisons should select a set of goals that are relevant for both the disease and the technology being assessed as part of the HTA process.

Step 3: Measuring performance. Once the goals have been selected, the researchers, patient liaisons, and additional stakeholders appropriate for the MCDA process should gather data on impact of the technology alternatives on the goals. Technologies may have different impacts on different goals, so the goals should be added to the "performance matrix" to allow comparison across the alternatives. For new technologies or for new applications of existing technologies, data on impact may not

be available. The stakeholder group should jointly determine potential impacts.

Step 4: Scoring alternatives. Once the goals have been added to the performance matrix, patient liaisons should review the previously collected data from the larger group of patients as part of Step 0 on the preferences for different levels of goal attainment. The patient liaisons will then summarize this data as it applies to the alternatives to score each alternative in how well it can help patients achieve their goals.

Step 5: Weighting criteria. Patient liaisons should summarize patients' weightings of the goals from Step 0 to determine which goals are most important and should be given more weight in the MCDA process.

Step 6: Calculating aggregate scores and **Step 7: Dealing with uncertainty.** We have not prioritized patient involvement in aggregation and uncertainty ratings, as these activities do not involve deliberation. However, with training, patient liaisons could be involved in these activities, and doing so might improve transparency of and trust in the procedures used to estimate value.

Step 8: Reporting and examination of findings. Once the outputs of the MCDA are produced, the patient liaisons and researchers should communicate them to the involved patients, gather feedback from the patients, and close the loop to encourage future participation in health care valuation exercises. We should be mindful of the complexities of the MCDA process and communicate findings in a way that does not require patients to learn the intricacies of the MCDA calculations.

How We Assessed Feasibility and Developed Recommendations

The feasibility of the above proposed approach was established through a project focusing on people with rheumatoid arthritis (RA). We chose RA as an ideal disease state in which to conduct a feasibility study of our Patient-Engaged Health Technology Assessment strategy. Obtaining patient input is particularly relevant for a disease state like RA where there is discrepancy between the outcomes included in clinical trials and value assessments, and the outcomes that patients care about most (Bingham, Alten, & De Wit, 2012; Orbai & Bingham, 2015). While some patient-important treatment goals are commonly captured (e.g., slowed or stopped disease progression), others are not. For example, while many patients rank symptomology such as fatigue and functional limits very highly, researchers often describe symptomology as "subclinical," and most choose routine blood monitoring to assess interventions in RA. Nevertheless, patient-important outcomes are gaining ground in RA

research and care, and patient-reported outcomes (PROs) are increasingly becoming a part of core outcome sets recommended for both clinical trials and routine documentation (Radner et al., 2018). The European Alliance of Associations for Rheumatology (EULAR) and Outcome Measures in Rheumatoid Arthritis Clinical Trials (OMERACT) are driving adoption of patient-important outcomes in clinical trials by developing and improving consensus for research priorities and outcome measures with direct participation from patient stakeholders (OMERACT Outcome Measures in Rheumatology, 2021; Smolen et al., 2017; Tugwell et al., 2007) and through direct research support via funding.

This project was comprised of three major activities. We formed a project Steering Committee to review the feasibility of using patient-important goals and inclusive deliberations in HTAs, and to assist with identifying a candidate set of goals for health interventions in RA for use in a feasibility test. We then conducted a survey of people living with RA and asked them to rate the relative importance of RA-specific goals that were identified by the Steering Committee and solicited open-ended input to collect goals from patients directly. Finally, we formed an Expert Panel to review the feasibility results and develop practical recommendations for scaling these goals for other settings and other patient populations, with a focus on how the methods could be applied to incorporating patient input into HTA. The practical recommendations reported here are intended to serve as a guide for wider use of the Patient-Engaged Health Technology Assessment Strategy.

Steering Committee

At the outset of this project, RAND recruited 10 stakeholders to join a Steering Committee to review the proposed strategy, aid in a feasibility assessment of its major steps, and inform the implementation of our patient survey. Stakeholders represented patients (n=3), consumer advocates (n=2), clinicians (n=1), researchers with expertise in RA patient engagement (n=2), and experts in methods for MCDA and patient preference assessment (n=2). The Steering Committee met twice in 2020. Steering Committee members reviewed five main features of the strategy:

1) Involving patient communities in identification of relevant outcomes for inclusion in HTA exercises
2) Ensuring full and inclusive representation of all patients by matching patient-level characteristics in HTA exercises with the patient communities that may use the intervention in question
3) Identifying patient-important outcomes by conducting a GAS exercise at scale via a survey or via data collection in clinical settings
4) Selecting a final set of patient-important outcomes by working with a smaller group of patients to rank goals identified in the GAS exercise
5) Working with patients in a deliberative exercise to complete the HTA, such as an

MCDA value measurement model.

After the second meeting, members were asked to complete a strategy feasibility rating in which they rated the feasibility of a series of statements, including "Patient goals can be collected via online surveys" and "MCDA is a useful method for establishing the value of new treatments," and provided comments about the strategy.

Patient Survey

The value of collecting information about treatment effectiveness and elements of treatment value directly from patients has been demonstrated in RA.[15] For instance, PROMIS® short forms have been found to capture meaningful improvement or worsening in key symptom features of RA (Bartlett, Gutierrez, et al., 2020), supporting the value of direct patient report in RA. Beyond the validity of PROs, engaging patients in their care by asking them to rate symptoms using PRO tools has also been found to improve the patient experience, including confidence in treatment decisions (Bartlett, De Leon, et al., 2020).

We worked with the project Steering Committee to develop and implement a survey to identify patient goals for RA treatment and rate the importance of the goals on a four-point scale. Goal statements were listed within four domains: symptom management, life impact, managing my RA, and treatment features. A final section entitled "other goals" allowed respondents to write in other responses. Respondents were also asked to provide demographic information and respond to five questions about their experiences living with RA (age at diagnosis, disease severity, etc.). We surveyed a total of 47 patients recruited through two RA patient organizations. Every respondent rated at least one goal as "Very Important," but there was variation in the importance of the different goals among respondents (Bartlett et al., 2022).

Expert Panel

Five of the nine Expert Panel members had also participated in the Steering Committee, providing continuity before and after the Patient Survey was fielded. Four of the nine members, including two new patient representatives and two additional methodologists, were recruited to add new perspectives. The Expert Panel met in early 2022 to review the results of the Patient Survey and provide qualitative comments on the feasibility of the methodology.

Feasibility Findings and Key Considerations

We have synthesized the major feasibility findings from review by the Steering Committee before the survey was fielded and Expert Panel after the survey was fielded. Overall, Steering Committee members and expert panelists expressed that the process was feasible and that generating and using a patient-centered goal inventory in HTA will improve the patient-centeredness of health care valuation. They agreed that the feasibility of the method is

paramount to its use in practice, and they agreed that there was a need to clearly identify who is involved in all aspects of the process, when the patient community is involved and how, and that the method must be transparent and easily understood. To further explore the feasibility of specific steps in the process, we have grouped other feasibility findings into the previously described steps in the MCDA process.

Step 0: Develop goal inventory. Panelists noted that for many therapeutic areas outcome measures already exist that can serve as the foundation for the goal inventory, with supplementation through literature reviews emphasizing treatment effectiveness. The goal inventory can also be developed or supplemented through patient surveys, like the one used in the pilot for this project. The initial survey can include patient-based importance ratings, and the prioritized goals identified via those ratings can be used as the goal set for further use in the MCDA exercise. For many therapeutic areas patients can be asked to identify additional goals beyond those included in the initial survey as one way to ensure more complete coverage of important outcomes from the perspective of patients. Collection of goals along with importance ratings using this method can be an efficient way to generate the foundation for the subsequent steps.

Research teams, including patient advocates, patients, and clinicians, can periodically review and update these goals. The universe of outcomes and goals should reflect what is important to the representative patient populations in the real world, not just those included in clinical trials. Outcomes important to the broader population for which HTA results are intended to apply must be captured. The interdependence and correlations among goals should be monitored, and practitioners should avoid counting the same concepts as independent concepts just because they are expressed slightly differently. However, unrelated goals may be collinear; collinearity alone would not be a reason to drop goals from the set.

The panelists also pointed out that identifying goals and valuing goals are sequential procedures. In the first, patients may work from a library of goals and add those they do not see articulated. In the second, patients rate the selected goals.

Step 1: Defining the decision problem. Both the Steering Committee and Expert Panel members felt that existing patient and consumer health networks could support this work. However, ensuring *broad* representation of patients in MCDA work is

> *"There's value added in bringing a broader perspective to the decision."*
>
> Expert Panel member

important to understanding how patient goals may vary across key patient-level clinical and sociodemographic characteristics. Both groups also agreed that liaising patients are an important element of the strategy, pointing out that working with well-known community advocates can enhance trust and result in richer goal elicitation. Additionally, they recommended that objectives for patient engagement should be clarified at the outset. Language and terminology should be clear to all participating patients and should be consistent throughout the process. Addressing differences in health literacy and explaining the goals and steps of MCDA within the patient community is required.

Step 2: Selecting and structuring criteria. Goals selected should be important to patients but also relevant for the specific MCDA exercise being conducted. Patient liaisons should be aware of the ways that a new treatment alternative being evaluated would provide value for patients and select goals accordingly.

Step 3: Measuring performance. The panelists noted that goal sets need to be constructed carefully, with clinician as well as patient input. Patient history may explain differences in performance of technology alternatives on goals. For example, there are important differences between patients diagnosed prior to versus after the introduction of biologics as a treatment option for RA. Further, the extent of irreversible joint damage may influence a patient's goal ratings. Finally, patients who have experienced a change of symptoms after switching treatments may rate goals differently from those who never changed therapies or who experienced steady outcomes.

> *"In developing and validating instruments for quality of life and RA the context is so important here. How you rank these items depends on how you're feeling right now, and a lot on your experience."*

To measure the impact of technologies on goals, existing data sources should be used if available, including outcomes data collected through trials. Outcomes from trials could be mapped to some outcomes identified as important through this strategy. Some trial endpoints on pain and functioning, for example, may overlap with patient-important goals or outcomes. Some outcomes important to patients will not be captured in clinical trial data, and panelists noted that many of these outcomes may not be collected anywhere, even within real-world evidence datasets, so additional data collection by researchers may be needed.

Step 4: Scoring alternatives. The Steering Committee agreed that people with lived experience of the disease should inform all criteria and weights. Patient goals can be collected at scale via online surveys.

Step 5: Weighting criteria. The panelists agreed that it was feasible to translate goal ratings into criteria weights for MCDA. Patient liaisons using the goal inventory from Step 0 can conduct the weighting exercise. This is because GAS, as a method of direct patient preference collection, avoids researcher interpretation of what matters to patients. All would require methods training to be familiar with the rationale and steps involved in the process. Some goals, such as certain types of physical functioning, are likely to be applicable to patients with a variety of health conditions and other characteristics, while other goals may vary across conditions or across other patient characteristics.

> *"You need both content AND patient engagement experts AND patient research partners to achieve optimal outcomes."*
>
> Steering Committee member

Step 6: Calculating aggregate scores and Step 7: Dealing with uncertainty. While patients could participate in aggregation and uncertainty ratings, the proposed process is limited to the prior steps for collection and use of patient input. Future iterations, once feasibility of the patient-engaged deliberation in Steps 1 through 5 is established, could involve patients being trained in these technical steps.

Step 8: Reporting and examination of findings. Panelists felt that once the MCDA process is complete, establishing a link between patient-centered goals and value of interventions may improve the translation of HTA in health decision-making back to patient communities, and supporting HTA with a patient-led method of goal elicitation and ranking may build credibility of health care valuation. Panelists also highlighted the need to communicate the measured patient goals in peer-reviewed publications and using other dissemination approaches. Ultimately, this strategy could inform endpoints in clinical trials and broaden the outcome set collected in efficacy trials. Panelists felt there is an opportunity to build on momentum across different organizations now to ensure that patient-generated data are included.

Above all, making a case for the value of patient-important outcomes is an important step that must occur prior to beginning the MCDA or other HTA deliberative procedure. Clinicians

may not view specific goals as important but may be persuaded when presented with data that patients view the goals as important. An example from oncology relates to frequency of administration. Clinicians may not value whether a treatment requires one or two administrations, but for patients that difference can be important, and this method would highlight these important aspects of patient-centered value.

Practical Recommendations for Implementing the Patient-Engaged Health Technology Assessment Strategy

The Expert Panel recommended following practical steps to make future health technology assessments more patient-centered and inclusive. The input from the Steering Committee, patient surveys, and Expert Panel yielded the following recommendations to guide patient engagement in HTA:

1. **Set clear objectives for patient inclusion**: Ensure that the types of decisions to be addressed are clear to stakeholders involved in the HTA process, and that the context in which the information collected from stakeholders will be used is clear. Relatedly, the purpose of a goal attainment approach for HTA use is to provide a means of obtaining patient-important goals. While this approach can also be used to quantify change in outcomes over time, doing so is not necessary for use of GAS for HTA.
2. **Address representativeness of patient data**: Obtain information from patients about their clinical context, including disease severity. Where a patient is in their "patient journey" influences their goals. The process should ensure that recruitment and involvement includes patients with relevant characteristics specific to the disease (i.e., disease severity, stage, experience with other therapies). If the initial goal inventory has a representative patient sample, it will allow us to examine the goals and priorities for a subset of patients based on the target patient population in the decision problem. Constraints on trial sample selection can be overcome somewhat by ensuring appropriate representativeness among patients participating in deliberation exercises. For some conditions (e.g., Alzheimer's disease, amyotrophic lateral sclerosis), patient caregivers or other proxies may need to be engaged as part of this process. One participant noted that some of the relevant data may be collected through clinical trials already. However, trial samples may not be optimally representative on key variables for the purposes of data use for HTA. One of the strengths of the Patient-Engaged Health Technology Assessment Strategy is addressing characteristics of the patients involved in the HTA process. The expectation is that data collection related to goals or outcomes of interest may be required to enhance data obtained from patients recruited to clinical trials.
3. **Ensure methods are rigorous**: Assemble evidence on psychometric performance of goal collection methods. Correlation between goals will impact results and should be

addressed prior to the deliberative processes, either by pruning goals to reduce those with high correlations or otherwise mitigating impact of collinearity. Once collected, goal sets require further analysis to examine magnitude of inter-goal correlation, by different populations and patient types. However, even if two goals are collinear, different technology alternatives may have different effects on these goals. Because the goals framework offers a way to broaden outcome considerations in HTA, goals should not be deleted from deliberation solely due to correlation with other goals.

4. **Communicate rationale for methods choices at different stages of the HTA process**. The goals framework allows for integrating the patient view in different ways. Goals can be used as criteria for MCDA and ranking of goals can also be used to aid with weighting exercises. Different audiences will need clarity on the purpose of method used and information collected at different steps.

In the United States there is a new opportunity to create ethically grounded and methodologically sound assessments of the value of health technologies. We can build from the decades of methods and experience with HTA from around the world. Methods for including people with relevant lived experience have evolved over that time. Patients, their families, and their caregivers can be included in determining treatment value, no longer only including researchers, health economists, and regulators in these decisions. Doing so enriches the information and strengthens the decisions that affect individuals and society.

Abbreviations

EULAR	European Alliance of Associations for Rheumatology
GAS	goal attainment scaling
HTA	health technology assessment
IVI	Innovation and Value Initiative
MCDA	multi-criteria decision analysis
NICE	National Institute for Health and Care Excellence
OMERACT	Outcome Measures in Rheumatoid Arthritis Clinical Trials
PRO	patient-reported outcome
QALY	quality-adjusted life year
RA	rheumatoid arthritis

References

Bartlett, S. J., Bingham, C., Predmore, Z., Concannon, T., Chen, E., Schrandt, S., . . . Frank, L. (2022). Testing a New Approach to Identify and Assess Patient-Valued Treatment Goals in Rheumatoid Arthritis (RA): A Patient-Engaged Healthcare Valuation Strategy Paper presented at the EULAR, Copenhagen, Denmark.

Bartlett, S. J., De Leon, E., Orbai, A.-M., Haque, U. J., Manno, R. L., Ruffing, V., . . . Leong, A. (2020). Patient-reported outcomes in RA care improve patient communication, decision-making, satisfaction and confidence: qualitative results. Rheumatology, 59(7), 1662-1670.

Bartlett, S. J., Gutierrez, A. K., Andersen, K. M., Bykerk, V. P., Curtis, J. R., Haque, U. J., . . . Bingham III, C. O. (2020). Identifying Minimal and Meaningful Change in PROMIS® for Rheumatoid Arthritis: Use of Multiple Methods and Perspectives. Arthritis care & research.

Bingham, C. O., Alten, R., & De Wit, M. P. (2012). The importance of patient participation in measuring rheumatoid arthritis flares. In (Vol. 71, pp. 1107-1109): BMJ Publishing Group Ltd.

Devlin, N. J., Shah, K. K., Mulhern, B. J., Pantiri, K., & van Hout, B. (2019). A new method for valuing health: directly eliciting personal utility functions. The European Journal of Health Economics, 20(2), 257-270.

Epstein, R. M., & Street, R. L. (2011). The values and value of patient-centered care. In (Vol. 9, pp. 100-103): Annals Family Med.

Facey, K. M., & Single, A. N. (2017). Patient input to HTA. In Patient involvement in health technology assessment (pp. 67-79): Springer.

Fraenkel, L., Nowell, W. B., Michel, G., & Wiedmeyer, C. (2018). Preference phenotypes to facilitate shared decision-making in rheumatoid arthritis. Annals of the rheumatic diseases, 77(5), 678-683.

Frank, L., Basch, E., Selby, J. V., & Institute, P.-C. O. R. (2014). The PCORI perspective on patient-centered outcomes research. Jama, 312(15), 1513-1514.

Frank, L., & Concannon, T. (2019). Here to represent: Optimizing representativeness and enhancing equity through patient-engaged healthcare valuation. Am J Manag Care, November Online Supplement. Retrieved from https://www.ajmc.com/journals/supplement/2019/optimizing-representativeness-patient-engaged-healthcare/optimizing-representativeness-patientengaged-healthcare-valuation

Garrison, L. P., Jansen, J. P., Devlin, N. J., & Griffin, S. (2019). Novel approaches to value assessment within the cost-effectiveness framework. Value in Health, 22(6), S12-S17.

Hansen, P., & Devlin, N. (2019). Multi-criteria decision analysis (MCDA) in healthcare decision-making. In Oxford Research Encyclopedia of Economics and Finance.

Jennings, L. A., Ramirez, K. D., Hays, R. D., Wenger, N. S., & Reuben, D. B. (2018). Personalized goal attainment in dementia care: measuring what persons with dementia and their caregivers want. Journal of the American Geriatrics Society, 66(11), 2120-2127.

Kiresuk, T. J., & Sherman, R. E. (1968). Goal attainment scaling: A general method for evaluating comprehensive community mental health programs. Community mental health journal, 4(6), 443-453.

Kiresuk, T. J., Smith, A., & Cardillo, J. E. (2014). Goal attainment scaling: Applications, theory, and measurement: Psychology Press.

Lakdawalla, D. N., Doshi, J. A., Garrison Jr, L. P., Phelps, C. E., Basu, A., & Danzon, P. M. (2018). Defining elements of value in health care—a health economics approach: an ISPOR Special Task Force report [3]. Value in Health, 21(2), 131-139.

Marsh, K., Goetghebeur, M., Thokala, P., & Baltussen, R. (2017). Multi-criteria decision analysis to support healthcare decisions (Vol. 10): Springer.

O'Rourke, B., Oortwijn, W., & Schuller, T. (2020). The new definition of health technology assessment: A milestone in international collaboration. International Journal of Technology Assessment in Health Care, 36(3), 187-190.

OMERACT Outcome Measures in Rheumatology. (2021). Patient Research Partner Home Page. 2021(April 14). Retrieved from https://omeractprpnetwork.org

Oortwijn, W., Husereau, D., Abelson, J., Barasa, E., Bayani, D. D., Santos, V. C., . . . Kieslich, K. (2022). Designing and Implementing Deliberative Processes for Health Technology Assessment: A Good Practices Report of a Joint HTAi/ISPOR Task Force. International Journal of Technology Assessment in Health Care, 38(1).

Orbai, A.-M., & Bingham, C. O. (2015). Patient reported outcomes in rheumatoid arthritis clinical trials. Current rheumatology reports, 17(4), 1-10.

Perfetto, E. M. (2018). ISPOR's initiative on US value assessment frameworks: a missed opportunity for ISPOR and patients. Value in Health, 21(2), 169-170.

Radner, H., Chatzidionysiou, K., Nikiphorou, E., Gossec, L., Hyrich, K. L., Zabalan, C., . . . Burmester, G. R. (2018). 2017 EULAR recommendations for a core data set to support observational research and clinical care in rheumatoid arthritis. Annals of the rheumatic diseases, 77(4), 476-479.

Rasburn, M., Livingstone, H., & Scott, S. E. (2021). Strengthening patient outcome evidence in health technology assessment: a coproduction approach. International Journal of Technology Assessment in Health Care, 37.

Sanders, G. D., Neumann, P. J., Basu, A., Brock, D. W., Feeny, D., Krahn, M., . . . Prosser, L. A. (2016). Recommendations for conduct, methodological practices, and reporting of cost-effectiveness analyses: second panel on cost-effectiveness in health and medicine. Jama, 316(10), 1093-1103.

Scott, A. M., & Wale, J. L. (2017). Patient advocate perspectives on involvement in HTA: an international snapshot. Research Involvement and Engagement, 3(1), 1-17.

Smolen, J. S., Landewé, R., Bijlsma, J., Burmester, G., Chatzidionysiou, K., Dougados, M., . . . Van Vollenhoven, R. (2017). EULAR recommendations for the management of rheumatoid arthritis with synthetic and biological disease-modifying antirheumatic drugs: 2016 update. Annals of the rheumatic diseases, 76(6), 960-977.

Thokala, P., Carlson, J. J., & Drummond, M. (2020). HTA'd in the USA: A Comparison of ICER in the United States with NICE in England and Wales. Journal of Managed Care & Specialty Pharmacy, 26(9), 1162-1170.

Thokala, P., Devlin, N., Marsh, K., Baltussen, R., Boysen, M., Kalo, Z., . . . Watkins, J. (2016). Multiple criteria decision analysis for health care decision making—an introduction: report 1 of the ISPOR MCDA Emerging Good Practices Task Force. Value in Health, 19(1), 1-13.

Tugwell, P., Boers, M., Brooks, P., Simon, L., Strand, V., & Idzerda, L. (2007). OMERACT: an international initiative to improve outcome measurement in rheumatology. Trials, 8(1), 1-6.